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**Bloom et al.**

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(54) **METHODS FOR SUSTAINED AND  
REGULATABLE GENE EXPRESSION USING  
VIRAL BASED EXPRESSION VECTORS**

(71) Applicant: **University of Florida Research  
Foundation, Inc.**, Gainesville, FL (US)

(72) Inventors: **David C. Bloom**, Gainesville, FL (US);  
**Antonio L. Amelio**, Pittsboro, NC (US)

(73) Assignee: **UNIVERSITY OF FLORIDA  
RESEARCH FOUNDATION, INC.**,  
Gainesville, FL (US)

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(58) **Field of Classification Search**

None

See application file for complete search history.

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*Primary Examiner* — Catherine S Hibbert

(74) *Attorney, Agent, or Firm* — Haynes and Boone, LLP

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**ABSTRACT**

Disclosed are genetic expression cassettes, and vectors com-  
prising them useful for the delivery of isolated nucleic acid  
segments including those expressing or encoding one or  
more selected therapeutic constructs (including, without  
limitation, therapeutic peptides, polypeptides, ribozymes, or  
catalytic RNA molecules), to one or more selected cells or  
tissues of a vertebrate animal. Methods employing the  
disclosed genetic constructs in the development of gene  
therapy-based viral vector systems are also disclosed. The  
expression cassettes and viral vectors disclosed herein pro-  
vide new tools for methods of treating mammalian, and in  
particular, human diseases, disorders, and/or dysfunctions.  
The disclosed compositions and methods find particular  
utility in a variety of investigative, diagnostic, and thera-  
peutic regimens, including, for example, in the treatment or  
amelioration of symptoms of a variety of mammalian, and  
particularly, human conditions.

**20 Claims, 12 Drawing Sheets**